RARE i

THE CANADIAN FORUM FOR
RARE DISEASE INNOVATORS

# Pre-Budget Submission
# 2021-22

Finance Canada

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## RAREi Recommendations

***1 –*** That the federal government accelerate its plans to invest $1 billion over two years to improve access to rare disease medicines.

***2 –*** That the federal government ensure that Canada is considered a preferred launch country for rare disease treatments, starting with the removal of the economic factors from the *Patented Medicines Regulations*.

***3 –*** The federal government should support holistic rare disease strategies to improve access to diagnosis, care and treatments for all Canadians with rare diseases.

## About RAREi

RAREi is an informal network of research-based bio pharmaceutical innovators committed to monitoring, responding and shaping policy issues in the Canadian rare disease environment. The members of RAREi are Akcea Therapeutics Canada, Alexion Pharma Canada Corp., Amicus Therapeutics, Inc., Biogen Canada Inc., Biomarin Pharmaceutical Inc., Horizon Therapeutics Canada, Ipsen Biopharmaceuticals Canada Inc., Mitsubishi Tanabe Pharma Canada Inc., Recordati Rare Diseases Canada Inc., Sanofi Genzyme, a division of sanofi-aventis Canada Inc., Sobi Canada Inc., Ultragenyx Pharmaceutical and Vertex Pharmaceuticals (Canada) Inc. For more information, see www.rarei.ca.

## Recommendations in Context

### *Recommendation 1* – That the federal government accelerate its plans to invest $1 billion over two years to improve access to rare disease medicines.

In Budget 2019, the government announced that "Special consideration is required to ensure a nationally consistent approach for [rare disease] medications," and committed $500 million per year "to help Canadians with rare diseases access the drugs they need" starting in 2022-23.[1] The promise to develop a national rare disease strategy was reiterated in the October 2020 Speech from the Throne and again in the November 2020 Economic update.

In January 2021, Health Canada launched the National Strategy for High-Cost Drugs for Rare Diseases consultation.[2] RAREi supports this initiative and is eager to collaborate meaningfully alongside patient groups, researchers, physicians, and all other relevant stakeholders in developing a national strategy for drugs for rare diseases. We recommend Health Canada move forward with the project as quickly as possible, and co-develop the strategy with the stakeholders mentioned above. There is a critical need to get this right and the best way to ensure buy-in from affected parties is to give them the opportunity to co-create it.

Rapidly evolving research and technological advances are leading to impressive breakthroughs for rare disease patients, most of whom struggle for years to obtain an accurate diagnosis only to find no reasonable treatment options are available. The recent emergence of many ground-breaking new therapies has offered hope for the future, but this is significantly tempered by Canada's existing policy framework and pharmaceutical review and approval processes.

A February 2020 *CMAJ* article outlined the many hurdles faced by Canadian rare disease patients in accessing available medicines, pointing out the barriers at each stage before a new medicine is listed on a formulary. Even then, restrictive criteria often derail attempts to receive treatment.[3] According to the Canadian Organization for Rare Disorders, 78% of patients with rare diseases experience challenges accessing needed medicines, with 70% facing delays and 58% denied access because of cost.[4]

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[1] Government of Canada, Budget 2019: Investing in the Middle Class, p. 62, March 19, 2019: https://budget.gc.ca/2019/docs/plan/budget-2019-en.pdf.
[2] Health Canada, January 27, 2021: https://www.canada.ca/en/health-canada/programs/consultation-national-strategy-high-cost-drugs-rare-diseases-online-engagement/discussion-paper.html.
[3] Rawson N and Lawrence D, *Patient Access to Essential Rare Disorder Drugs: The Long and Winding Road*, CMAJ Blogs, February 12, 2020: https://cmajblogs.com/patient-access-to-essential-rare-disorder-drugs-the-long-and-winding-road/#more-6843.
[4] Canadian Organization For Rare Disorders, Submission to House of Commons Standing Committee on Health, October 30, 2018: http://www.raredisorders.ca/content/uploads/FINAL-CORD-Brief_Barriers-to-accessing-treatments-for-rare-disorders_Oct-30-2018.pdf

Unlike most developed countries, Canada has no national rare disease treatment policy and payers are reluctant to reimburse them in the absence of national or provincial strategies.

Payers' are primarily concerned about their relatively higher public (list) prices. However, an evidence-based analysis found that non-oncology-related orphan medicines represented only 1.9% of total public medication expenditure in Canada in 2019, a percentage that is expected to increase to only 6.5% by 2025 for a total estimated spend of $1.39 billion. This estimate is likely far above actual spending, given that savings to public payers via product listing agreements were not included. The analysis concluded that public expenditure for rare disease treatments is minimal in comparison to other important expenditures.[5]

In sum, immediate federal support, both financial and organizational, is needed to ensure patient access to new rare disease medicines. RAREi urges the federal government to implement its rare disease budget commitments starting in 2021.

### ***Recommendation 2*** **– That the federal government ensure that Canada is considered a preferred launch country for rare disease treatments, starting with the removal of the economic factors from the *Patented Medicines Regulations*.**

The federal Liberal government signaled its intention to address pharmaceutical pricing issues in its election platform during the 2015 general election. In May 2017, then Health Minister Jane Philpott announced an ambitious consultation with the objective to implement a sweeping overhaul of the Patented Medicine Prices Review Board (PMPRB) to "modernize" its ability to address "increased drug spending" and respond to the "emergence of very expensive drugs designed to treat small numbers of people, often with very hefty price tags." The main changes would be 1- updating the basket of comparator countries which PMPRB uses for pricing benchmarks, 2- introducing pharmacoeconomic factors (usually reserved for health technology assessment) and 3- periodically revising maximum price thresholds down over the life cycle of a medicine.

Despite several rounds of consultations and thousands of concerned and constructive stakeholder submissions, a setback in both the Federal Court and the Quebec Superior Court around the obligations to report third party rebates, and three separate health ministers in five years, the PMPRB guidelines and the entire reform package are set to go into effect on July 1, 2021.

In August 2019, amendments were made to the *Patented Medicines Regulations* (PMRs). The reforms now give the PMPRB new powers to implement aggressive price controls,[6] including the application of new economic factors, leading to steep price reductions for almost all patented medicines in Canada. It is expected that public list prices for all patented medicines will fall by 15% on average. Medications deemed high-cost or high-volume will be subject to further 35-65% price reductions. These cuts are significantly higher than original Health Canada projections, creating unprecedented business uncertainty.

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[5] Forte L et al., *The Current and Future Costs of Orphan Drugs in Canada - A Public Payer Budget Impact Analysis*, Patient Access Solutions, 2019 ISPOR Europe Poster, November 2019: https://www.ispor.org/heor-resources/presentations-database/presentation/euro2019-3122/96632. See Patient Access Solution's analysis in RAREi's submission to the PMPRB https://www.canada.ca/content/dam/pmprb-cepmb/documents/consultations/draft-guidelines/submission-received/june2020/June%202020%20submission_RAREi_EN.pdf.

[6] *Patented Medicines Regulations,* Canada Gazette II, August 21 2019: http://gazetteducanada.gc.ca/rp-pr/p2/2019/2019-08-21/html/sor-dors298-eng.html

These are the most significant changes to the pharmaceutical pricing environment in more than 30 years. The new regulations are a major departure from the current approach taken by the PMPRB, they extend beyond the bounds of its mandate and they will alter the landscape of access to medicines in Canada.

For instance, research shows that Canada's status as an early market for new medicines[7] is at risk, which also threatens access to global clinical trials.[8] This means Canadians would not have prompt access to new medicines, causing poorer health outcomes and disruptions in provincial health systems.[9] A recent industry leaders' survey confirmed those findings, revealing unanimity regarding the negative implications.[10]

In fact, uncertainty is already affecting access to new medicines and research. One study found new Canadian medicine launches dropped dramatically recently, corresponding with the timing of the PMPRB reforms. Most medicines launched globally in 2018 but not yet commercialized in Canada are for rare diseases and cancer.[11] For phase III/IV trials, the number in the first half of 2020 decreased 28% for oncology trials and 25% for non-oncology trials from the average for the same period in the previous five years.[12]

This suggests that the reforms will result in fewer breakthrough medicines – including vaccines and treatments being developed for COVID-19 – to cover under any future national pharmacare or rare disease strategy. That is the opposite of the federal government's access to rare disease medicines goal. They will also negatively impact the government's goal of doubling the size of the health and biosciences sector by 2025.[13]

***Recommendation 3*** **– The federal government should support holistic rare disease strategies to improve access to diagnosis, care and treatments for all Canadians with rare diseases.**

Part of the Budget 2019 rare disease commitment was a pledge to work with stakeholders and other governments "to build a coordinated strategy for gathering and evaluating evidence on high-cost drugs for rare diseases, improve the consistency of decision-making and access across the country, negotiate prices with drug manufacturers, and ensure that effective treatments reach the patients who need them."[14] In doing so, it promised to be guided by the recommendations of the Advisory Council on the Implementation of National Pharmacare (ACINP).

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[7] Skinner B., *Consequences of over-regulating the prices of new drugs in Canada*, Canadian Health Policy Institute, 2018 : https://www.canadianhealthpolicy.com/products/consequences-of-over-regulating-the-prices-of-new-drugs-in-canada.html.

[8] Skinner B., *Patented drug prices and clinical trials in 31 OECD countries 2017: implications for Canada's PMPRB*, Canadian Health Policy Institute, 2019: https://www.canadianhealthpolicy.com/products/patented-drug-prices-and-clinical-trials-in-31-oecd-countries-2017--implications-for-canada---s-pmprb-.html?buy_type=.

[9] Skinner B., *Consequences of over-regulating the prices of new drugs in Canada*, Canadian Health Policy Institute, 2018 : https://www.canadianhealthpolicy.com/products/consequences-of-over-regulating-the-prices-of-new-drugs-in-canada.html; and Ernst & Young report, *An assessment of Canada's current and potential future attractiveness as a launch destination for innovative medicines*, 2019: http://innovativemedicines.ca/wp-content/uploads/2019/02/2019_01_29_-IMC_PhRMA_LaunchSequencing_vFINAL3.pdf.

[10] Research Etc., Survey for Life Sciences Ontario, January 2021: https://lifesciencesontario.ca/wp-content/uploads/2021/01/Impact-of-Health-Canada-Pricing-Reform-FINAL-Report-Jan-21-2021.pdf.

[11] Life Sciences Ontario Webinar: *New Medicine Launches: Canada in a Global Context*, June 2020: https://lifesciencesontario.ca/wp-content/uploads/2020/06/EN_LSO_Global-Launch-Benchmarking_Webinar-June22-20_Final.pdf.

[12] Rawson, N, Clinical Trials in Canada: Worrying Signs that Uncertainty Regarding PMPRB Changes will Impact Research Investment, Canadian Health Policy, January 2021. Toronto: Canadian Health Policy Institute: https://www.canadianhealthpolicy.com/products/clinical-trials-in-canada--worrying-signs-that-pmprb-changes-will-impact-research-investment.html.

[13] Report from Canada's Economic Strategy Tables: Health and Biosciences, September 2018: https://www.ic.gc.ca/eic/site/098.nsf/eng/00025.html.

[14] Government of Canada, Budget 2019: Investing in the Middle Class, p. 62, March 19, 2019: https://budget.gc.ca/2019/docs/plan/budget-2019-en.pdf.

The ACINP called for "a distinct national process for providing fair, consistent, timely and evidence-based access" to rare disease treatments and a "distinct pathway" for their review and approval led by a national expert panel that "would work with patients and their care teams, (and others) reviewing individual cases to determine whether a particular drug should be funded for a particular individual."[15]

RAREi supports these government commitments but in order to expand access to effective treatments, a comprehensive and distinct national rare disease treatment coverage program is required that enhances all elements of the Canadian process, including regulatory, pricing and access policies and funding. Each stage needs to be streamlined and customized to address the specific needs of patients with rare diseases rather than the current broad population-based approach. As the 2021 National Strategy for High-Cost Drugs for Rare Diseases consultation begins, RAREi believes the following changes should be included:

- **A regulatory framework that incents the development and commercialization of rare disease therapies.** This would include a definition of rare disease, an orphan product designation process, market exclusivity, research promotion funds, tax incentives and regulatory submission fee reductions.
- **Pricing tests and tools that do not rely on cost-effectiveness assessments or have a disproportionate impact on rare disease treatments.** Federal government patented medicine pricing reforms would introduce a huge access barrier by requiring regulated price reductions of as much as 65% for rare disease medicines[16] and a disproportionate effect on rare disease treatments.[17] They need to be reconsidered.
- **Health Technology Assessment reviews should include processes, criteria and standards appropriately suited for rare diseases.** Health technology assessment reviews that are undertaken at the pan-Canadian provincial and private payer levels currently rely on evaluation methodologies that have been shown to be biased against treatments that serve smaller patient populations. These methods can be enhanced by formally incorporating specialized clinician expertise, patient values, more frequent reliance on observational studies and real-world evidence (RWE) beyond randomized clinical trials, and recognizing the reality of smaller study sizes and the requirement for higher per patient prices for these treatments.
- **Reimbursement processes, including negotiation and funding decisions, that provide timely and equitable access to rare disease treatments.** Public drug plan funding should implement measures used elsewhere, such as allowing reimbursement at the time of regulatory approval, increased use of pay-for-performance, managed access programs and RWE to address clinical and economic uncertainties without delaying patient access.
- **Investments in RWE research, infrastructure and policy development.** RWE can fill critical clinical gaps and increase certainty for public drug plans, payers and manufacturers, especially for orphan therapies. Health Canada is already leading efforts to expand RWE use, and the federal government should continue to support a national RWE framework[18] by investing in research funding, patient registries and centres of clinical expertise, as well as pursuing enhanced regulatory reviews that support manufacturer data development submitted in regulatory applications.

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[15] Final Report of the Advisory Council on the Implementation of National Pharmacare, *A Prescription for Canada: Achieving Pharmacare for All*, September 24, 2019: https://www.canada.ca/en/health-canada/corporate/about-health-canada/public-engagement/external-advisory-bodies/implementation-national-pharmacare/final-report.html.

[16] Rawson, N., *Regulatory, Reimbursement, and Pricing barriers to accessing Drugs for Rare Disorders in Canada*, Fraser Institute, 2018: https://www.fraserinstitute.org/sites/default/files/barriers-to-accessing-drugs-for-rare-disorders-in-canada.pdf.

[17] See also Rawson N., *New Patented Medicine Regulations in Canada: Case Study of a Manufacturer's Decision-Making about Regulatory Submission for a Rare Disorder Treatment*, Canadian Health Policy Institute, October 2018: https://www.canadianhealthpolicy.com/products/new-patented-medicine-regulations-in-canada--case-study-of-a-manufacturer---s-decision-making.html.

[18] Institute for Health Economics (IHE), *Defining decision-grade real-world evidence and its role in the Canadian context: A design sprint* – Summary report, October 21, 2018: https://www.ihe.ca/events/past/conferences/ihe-capt-rwe/ihe-capt-rwe-about.

Several of these initiatives were elaborated in RAREi's recommendations to the House of Commons Standing Committee on Health for a rare disease study,[19] and some were highlighted in the ACINP final report.[20] Both of these committees recognized the need for a distinct approach to rare disease treatments.

[19] RAREi, *Unique approach needed: Addressing barriers to accessing rare disease treatments*, October 31, 2018: https://www.linkedin.com/feed/update/urn:li:activity:6556579888877363200. *See also* Standing Committee on Health, *Canadians Affected by Rare Diseases and Disorders: Improving Access to Treatment*, February 2019: https://www.ourcommons.ca/DocumentViewer/en/42-1/HESA/report-22/.

[20] Advisory Council on the Implementation of National Pharmacare, *A Prescription for Canada: Achieving Pharmacare for All*, June 12, 2019: https://www.canada.ca/en/health-canada/corporate/about-health-canada/public-engagement/external-advisory-bodies/implementation-national-pharmacare/final-report.html.